

## SEQUENCE LISTING

&lt;110&gt; Hildinger, Markus

<120> Decreasing gene expression in a mammalian subject in vivo via  
AAV-mediated RNAi expression cassette transfer

&lt;130&gt; 2

&lt;140&gt; US 10/604,340

&lt;141&gt; 2003-07-13

&lt;160&gt; 12

&lt;170&gt; PatentIn version 3.2

&lt;210&gt; 1

&lt;211&gt; 6437

&lt;212&gt; DNA

&lt;213&gt; Artificial

&lt;220&gt;

<223> sequence for recombinant adeno-associated viral vector, including  
plasmid backbone, with AAV2 internal terminal repeats that flank  
expression cassette; referred to as AAV2/2 CMV luciferase

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&lt;221&gt; CDS

&lt;222&gt; (1228)..(2883)

&lt;223&gt; luciferase cDNA

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Val Asp Ile Thr Tyr Ala Glu Tyr Phe Glu Met Ser Val Arg Leu Ala  
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Phe Ile Gly Val Ala Val Ala Pro Ala Asn Asp Ile Tyr Asn Glu Arg  
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Ser Lys Lys Gly Leu Gln Lys Ile Leu Asn Val Gln Lys Lys Leu Pro  
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Ile Ile Gln Lys Ile Ile Met Asp Ser Lys Thr Asp Tyr Gln Gly  
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Phe Gln Ser Met Tyr Thr Phe Val Thr Ser His Leu Pro Pro Gly Phe  
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Ala Leu Pro His Arg Thr Ala Cys Val Arg Phe Ser His Ala Arg Asp  
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Pro Ile Phe Gly Asn Gln Ile Ile Pro Asp Thr Ala Ile Leu Ser Val  
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Val Pro Phe His His Gly Phe Gly Met Phe Thr Thr Leu Gly Tyr Leu  
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Asp Leu Ser Asn Leu His Glu Ile Ala Ser Gly Gly Ala Pro Leu Ser  
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Lys Glu Val Gly Glu Ala Val Ala Lys Arg Phe His Leu Pro Gly Ile  
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<211> 6437

<212> DNA

<213> Artificial

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<223> sequence for recombinant adeno-associated viral vector, including plasmid backbone, with AAV2 internal terminal repeats that flank expression cassette; referred to as AAV2/5 CMV luciferase

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<222> (1228)..(2883)

<223> luciferase cDNA

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act ggt ctg cct aaa ggt gtc gct ctg cct cat aga act gcc tgc gtg Thr Gly Leu Pro Lys Gly Val Ala Leu Pro His Arg Thr Ala Cys Val			1878
205	210	215	
aga ttc tcg cat gcc aga gat cct att ttt ggc aat caa atc att ccg Arg Phe Ser His Ala Arg Asp Pro Ile Phe Gly Asn Gln Ile Ile Pro			1926
220	225	230	
gat act gcg att tta agt gtt gtt cca ttc cat cac ggt ttt gga atg Asp Thr Ala Ile Leu Ser Val Val Pro Phe His His Gly Phe Gly Met			1974
235	240	245	
ttt act aca ctc gga tat ttg ata tgt gga ttt cga gtc gtc tta atg phe Thr Thr Leu Gly Tyr Leu Ile Cys Gly Phe Arg Val Val Leu Met			2022
250	255	260	265
tat aga ttt gaa gaa gag ctg ttt ctg agg agc att cag gat tac aag Tyr Arg Phe Glu Glu Leu Phe Leu Arg Ser Leu Gln Asp Tyr Lys			2070
270	275	280	
att caa agt gcg ctg ctg gtg cca acc cta ttc tcc ttc ttc gcc aaa Ile Gln Ser Ala Leu Leu Val Pro Thr Leu Phe Ser Phe Phe Ala Lys			2118
285	290	295	

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tct ggt ggc gct ccc ctc tct aag gaa gtc ggg gaa gcg gtt gcc aag Ser Gly Gly Ala Pro Leu Ser Lys Glu Val Gly Glu Ala Val Ala Lys 315 320 325	2214
agg ttc cat ctg cca ggt atc agg caa gga tat ggg ctc act gag act Arg Phe His Leu Pro Gly Ile Arg Gln Gly Tyr Gly Leu Thr Glu Thr 330 335 340 345	2262
aca tca gct att ctg att aca ccc gag ggg gat gat aaa ccg ggc gag Thr Ser Ala Ile Leu Ile Thr Pro Glu Gly Asp Asp Lys Pro Gly Ala 350 355 360	2310
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<220>  
 <223> Synthetic Construct

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Tyr Ala Leu Val Pro Gly Thr Ile Ala Phe Thr Asp Ala His Ile Glu  
 35 40 45

Val Asp Ile Thr Tyr Ala Glu Tyr Phe Glu Met Ser Val Arg Leu Ala  
 50 55 60

Glu Ala Met Lys Arg Tyr Gly Leu Asn Thr Asn His Arg Ile Val Val  
 65 70 75 80

Cys Ser Glu Asn Ser Leu Gln Phe Phe Met Pro Val Leu Gly Ala Leu  
 85 90 95

Phe Ile Gly Val Ala Val Ala Pro Ala Asn Asp Ile Tyr Asn Glu Arg  
 100 105 110

Glu Leu Leu Asn Ser Met Gly Ile Ser Gln Pro Thr Val Val Phe Val  
 115 120 125

Ser Lys Lys Gly Leu Gln Lys Ile Leu Asn Val Gln Lys Lys Leu Pro  
 130 135 140

Ile Ile Gln Lys Ile Ile Ile Met Asp Ser Lys Thr Asp Tyr Gln Gly  
 145 150 155 160

Phe Gln Ser Met Tyr Thr Phe Val Thr Ser His Leu Pro Pro Gly Phe  
 165 170 175

Asn Glu Tyr Asp Phe Val Pro Glu Ser Phe Asp Arg Asp Lys Thr Ile  
 180 185 190

Ala Leu Ile Met Asn Ser Ser Gly Ser Thr Gly Leu Pro Lys Gly Val  
 195 200 205

Ala Leu Pro His Arg Thr Ala Cys Val Arg Phe Ser His Ala Arg Asp  
 210 215 220

Pro Ile Phe Gly Asn Gln Ile Ile Pro Asp Thr Ala Ile Leu Ser Val  
 225 230 235 240

Val Pro Phe His His Gly Phe Gly Met Phe Thr Thr Leu Gly Tyr Leu  
 245 250 255

Ile Cys Gly Phe Arg Val Val Leu Met Tyr Arg Phe Glu Glu Leu  
 260 265 270

Phe Leu Arg Ser Leu Gln Asp Tyr Lys Ile Gln Ser Ala Leu Leu Val  
 275 280 285

Pro Thr Leu Phe Ser Phe Phe Ala Lys Ser Thr Leu Ile Asp Lys Tyr  
 290 295 300

Asp Leu Ser Asn Leu His Glu Ile Ala Ser Gly Gly Ala Pro Leu Ser  
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Lys Glu Val Gly Glu Ala Val Ala Lys Arg Phe His Leu Pro Gly Ile  
 325 330 335

Arg Gln Gly Tyr Gly Leu Thr Glu Thr Thr Ser Ala Ile Leu Ile Thr  
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Pro Glu Gly Asp Asp Lys Pro Gly Ala Val Gly Lys Val Val Pro Phe  
 355 360 365

Phe Glu Ala Lys Val Val Asp Leu Asp Thr Gly Lys Thr Leu Gly Val  
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Asn Gln Arg Gly Glu Leu Cys Val Arg Gly Pro Met Ile Met Ser Gly  
 385 390 395 400

Tyr Val Asn Asn Pro Glu Ala Thr Asn Ala Leu Ile Asp Lys Asp Gly  
 405 410 415

Trp Leu His Ser Gly Asp Ile Ala Tyr Trp Asp Glu Asp Glu His Phe  
 420 425 430

Phe Ile Val Asp Arg Leu Lys Ser Leu Ile Lys Tyr Lys Gly Tyr Gln  
 435 440 445

Val Ala Pro Ala Glu Leu Glu Ser Ile Leu Leu Gln His Pro Asn Ile  
 450 455 460

Phe Asp Ala Gly Val Ala Gly Leu Pro Asp Asp Asp Ala Gly Glu Leu  
 465 470 475 480

Pro Ala Ala Val Val Val Leu Glu His Gly Lys Thr Met Thr Glu Lys  
 485 490 495

Glu Ile Val Asp Tyr Val Ala Ser Gln Val Thr Thr Ala Lys Lys Leu  
 500 505 510

Arg Gly Gly Val Val Phe Val Asp Glu Val Pro Lys Gly Leu Thr Gly  
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<212> DNA  
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<220>  
<223> sequence for recombinant adeno-associated viral vector, including plasmid backbone, with AAV2 internal terminal repeats that flank expression cassette; referred to as AAV2/5 U6 lucRI-1a

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<211> 3920  
<212> DNA  
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<220>  
<223> sequence for recombinant adeno-associated viral vector, including plasmid backbone, with AAV2 internal terminal repeats that flank expression cassette; referred to as AAV2/5 U6 lucRI-1b

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<210> 7  
<211> 3923  
<212> DNA  
<213> Artificial

&lt;220&gt;

<223> sequence for recombinant adeno-associated viral vector, including plasmid backbone, with AAV2 internal terminal repeats that flank expression cassette; referred to as AAV2/5 U6/U6 lucRIU6-3

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<210> 8  
<211> 3589  
<212> DNA  
<213> Artificial

&lt;220&gt;

<223> sequence for recombinant adeno-associated viral vector, including plasmid backbone, with AAV2 internal terminal repeats that flank expression cassette; referred to as AAV2/5 U6 lucRI-4(sense)

&lt;400&gt; 8

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<210> 9  
<211> 3589  
<212> DNA  
<213> Artificial

&lt;220&gt;

<223> sequence for recombinant adeno-associated viral vector, including plasmid backbone, with AAV2 internal terminal repeats that flank expression cassette; referred to as AAV2/5 U6 lucRI-4(antisense)

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 <211> 3617  
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 <213> Artificial

&lt;220&gt;

<223> sequence for recombinant adeno-associated viral vector, including plasmid backbone, with AAV2 internal terminal repeats that flank expression cassette; referred to as AAV2/2 U6 eGFPRI-1a

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&lt;210&gt; 11

&lt;211&gt; 3787

&lt;212&gt; DNA

&lt;213&gt; Artificial

&lt;220&gt;

&lt;223&gt; sequence for recombinant adeno-associated viral vector, including plasmid backbone, with AAV2 internal terminal repeats that flank expression cassette; referred to as AAV2/5 pol1 lucRI

&lt;400&gt; 11

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